Union Calendar No. 300

100TH CONGRESS
1ST SESSION

H. R. 3459

[Report No. 100–473]

To amend the Federal Food, Drug, and Cosmetic Act to revise the provisions respecting orphan drugs and for other purposes.

IN THE HOUSE OF REPRESENTATIVES

OCTOBER 9, 1987

Mr. WAXMAN introduced the following bill; which was referred to the Committee on Energy and Commerce

DECEMBER 10, 1987

Reported with an amendment, committed to the Committee of the Whole House on the State of the Union, and ordered to be printed

[Strike out all after the enacting clause and insert the part printed in italic]

[For text of introduced bill, see copy of bill as introduced on October 9, 1987]

A BILL

To amend the Federal Food, Drug, and Cosmetic Act to revise the provisions respecting orphan drugs and for other purposes.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SECTION 1. SHORT TITLE.

This Act may be cited as the "Orphan Drug Amendments of 1987".

SEC. 2. MARKET PROTECTION.

(a) LIMIT.—

(1) Section 527(a) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360cc(a)) is amended to read as follows:

"(a) Except as provided in subsection (b), if the Secretary—

"(1) approves an application filed under section 505(b) for a drug designated under section 526 for a rare disease or condition and the application is the first section 505(b) application approved for such drug for such disease or condition, then during the 7-year period beginning on the date the Secretary approved such first application—

"(A) the Secretary may approve for any person an application under section 505(b) (other than an application which includes a certification described in section 505(b)(2)) for such drug for such disease or condition, and

"(B) the Secretary may not approve—

"(i) an application under section 505(j), or

"(ii) an application which includes a certification described in section 505(b)(2)

for such drug for such disease or condition, for a person who is not the holder of an approved appli-

cation under section 505(b) for such drug for such disease or condition,

"(2) issues a certification under section 507 for a drug designated under section 526 for a rare disease or condition and the certification is the first certification issued under section 507 for such drug for such disease or condition, then during the 7-year period beginning on the date the Secretary issued such first certification—

"(A) the Secretary may approve for any person an application (other than an application described in subparagraph (B)) for a certification of such drug for such disease or condition under section 507, and

"(B) the Secretary may not issue a certification with respect to—

"(i) an abbreviated application filed under section 507, or

"(ii) an application filed under section 507 which includes investigations of the type described in section 505(b)(2)

for such drug for such disease or condition for a person who is not the holder of an approved certification under section 507 for such drug for such disease or condition, or

"(3) issues a license under section 351 of the Public Health Service Act for a drug designated under section 526 for a rare disease or condition and the license is the first license issued under such section 351 for such drug for such disease or condition, then during the 7-year period beginning on the date the Secretary issued such first license—

"(A) the Secretary may approve for any person an application (other than an application deseribed in subparagraph (B)) for a license under such section 351 for such drug for such disease or condition, and

"(B) the Secretary may not approve an application for a license for such drug for such disease or condition which is of the type which includes investigations of the type described in section 505(b)(2) for a person who is not the holder of a license issued under such section 351 for such drug for such disease or condition.".

(2) Section 527(b) of such Act (21 U.S.C. 360cc(b)) is amended—

(A) by striking out "505" the first place it appears and inserting in lieu thereof "505(b)", and

(B) by inserting after "issuance of the license" the following: "and notwithstanding subsection (a)".

(b) TECHNICALS.—

(1) Section 525(a) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360aa(a)) is amended by striking out "505" each place it appears and inserting in lieu thereof "505(b)".

(2) Section 526(a)(1)(A) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bb(a)(1)(A)) is amended by striking out "505" and inserting in lieu thereof "505(b)".

(3) Section 527 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360cc) is amended—

(A) by striking out the second comma after "507" the third time it appears in subsection (b), and

(B) by striking out "(1) The" in subsection (b)(1) and inserting in lieu thereof "(1) the".

SEC. 3. DESIGNATION AS AN ORPHAN DRUG.

(a) REQUEST.—Section 526(a)(1) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bb(a)(1)) is amended by adding after the first sentence the following: "A request for designation of a drug shall be made before the submission of an application under section 505(b) for the drug, the sub-

mission of an application for certification of the drug under section 507, or the submission of an application for licensing of the drug under section 351 of the Public Health Service Act.".

(b) DISCONTINUANCE.—Section 526 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bb) is amended by redesignating subsections (b) and (c) as subsections (c) and (d), respectively, and by adding after subsection (a) the following:

"(b) A designation of a drug under subsection (a) shall be subject to the condition that—

"(1) if an application was approved for the drug under section 505(b), a certificate was issued for the drug under section 507, or a license was issued for the drug under section 351 of the Public Health Service Act, the manufacturer of the drug will notify the Secretary of any discontinuance of the production of the drug at least one year before discontinuance, and

"(2) if an application has not been approved for the drug under section 505(b), a certificate has not been issued for the drug under section 507, or a license has not been issued for the drug under section 351 of the Public Health Service Act and if preclinical investigations or investigations under section 505(i) are being conducted with the drug, the manufacturer or

sponsor of the drug will notify the Secretary of any decision to discontinue active pursuit of approval of an application under section 505(b), approval of an application for certification under section 507, or approval of a license under section 351 of the Public Health Service Act.".

SEC. 4. FINANCIAL ASSISTANCE.

(a) MEDICAL DEVICES.—Section 5 of the Orphan Drug Act (21 U.S.C. 360ee) is amended—

(1) in subsection (a), by inserting "(1)" after "assist in" and by inserting before the period a comma and "(2) defraying the costs of developing medical devices for rare diseases or conditions", and

(2) in subsection (b)(2)—

(A) by inserting "(A) in the case of a drug," after "means" in the first sentence and by adding before the period in that sentence a comma and "(B) in the case of a medical device, any disease or condition that occurs so infrequently in the United States that there is no reasonable expectation that a medical device for such disease or condition will be developed without assistance under subsection (a)", and

(B) by striking out "under this subsection" in the last sentence and inserting in lieu thereof

"under section 526 of the Federal Food, Drug, and Cosmetic Act".

(b) MEDICAL FOODS.—Section 5 of the Orphan Drug Act (21 U.S.C. 360ee) is amended—

(1) in subsection (a) (as amended by subsection (a)), by inserting before the period a comma and "and (3) defraying the costs of developing medical foods for rare diseases or conditions",

(2) in subsection (b)(2) (as amended by subsection (a)), by inserting before the period at the end of the first sentence a comma and "and (C) in the case of a medical food, any disease or condition that occurs so infrequently in the United States that there is no reasonable expectation that a medical food for such disease or condition will be developed without assistance under subsection (a)", and

(3) by adding at the end of subsection (b) the following:

"(3) The term 'medical food' means a food which is formulated to be consumed or administered enterally under the supervision of a physician and which is intended for the specific dietary management of a disease or condition for which distinctive nutritional requirements, based on recognized scientific principles, are established by medical evaluation.".

(c) QUALIFICATION FOR ASSISTANCE.—Section 5(b) of the Orphan Drug Act (21 U.S.C. 360ee(b)) is amended—

(1) by striking out "and before the date on which an application with respect to such drug is submitted under section 505(b) or 507 of such Act or under section 351 of the Public Health Service Act" in paragraphs (1)(A)(ii) and (1)(B), and

(2) by striking out "and" at the end of subparagraph (A), by striking out the period at the end of subparagraph (B) and inserting in lieu thereof "; and", and by adding after subparagraph (B) the following:

"(C) in the case of a drug which has not been designated under section 526 of the Federal Food, Drug, and Cosmetic Act for a rare disease or condition but which the Secretary has reason to believe is a drug for a rare disease or condition, human clinical testing described in subparagraph (A)(i) and preclinical testing.".

(d) AUTHORIZATION.—Section 5(c) of the Orphan Drug Act (21 U.S.C. 360ee(c)) is amended to read as follows:

"(c) For grants and contracts under subsection (a) there are authorized to be appropriated $10,000,000 for fiscal year 1988, $12,000,000 for fiscal year 1989, $14,000,000 for fiscal year 1990.".

(d) STUDY.—The Secretary of Health and Human Services shall conduct a study to determine whether the application of subchapter B of chapter V of the Federal Food, Drug, and Cosmetic Act (relating to drugs for rare diseases and conditions) and section 28 of the Internal Revenue Code of 1986 (relating to tax credit) to medical devices or medical foods for rare diseases or conditions or to both is needed to encourage the development of such devices and foods. The Secretary shall report the results of the study to the Committee on Energy and Commerce of the House of Representatives and the Committee on Labor and Human Resources of the Senate not later than one year after the date of the enactment of this Act. For purposes of this section, the term "rare diseases or conditions" has the meaning prescribed by section 5 of the Orphan Drug Act (21 U.S.C. 360ee).

SEC. 5. NATIONAL COMMISSION ON ORPHAN DISEASES.

Section 4(n) of the Orphan Drug Amendments of 1985 (42 U.S.C. 236 note) is amended by striking out "September 30, 1987" and inserting in lieu thereof "February 1, 1989".

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